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An agency of the European Union

19 November 2026

Submission of comments on

Guideline on the clinical requirements for medicines intended for the treatment of thalassaemia

EMA/CHMP/HAEMWP/57392/2025

Name of organisation or individual*	General or Specific comment*	Line from* (line nr. or 0 for general comment)	Line to* (line nr. or 0 for general comment)	Comment and rationale
Institute for Quality and Efficiency in Health Care (IQWiG), Germany	General	0	0	We thank the EMA for the opportunity to comment on the guideline on the clinical requirements for medicines intended for the treatment of thalassaemia.
Institute for Quality and Efficiency in Health Care (IQWiG), Germany	General	0	0	We welcome the recommendations for clinical trials in the area of thalassaemia. The focus on sufficiently long randomised controlled trials with appropriate comparators and patient-centred outcomes, such as transfusion independence and health-related quality of life, encourages health technology developers to generate evidence relevant to both regulatory and health technology assessment (HTA) decision-making in a rare disease. Overall, IQWiG supports the draft guideline.

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